Demographic Data Summary for Novel Biological Approvals

January 1, 2023 – December 31, 2023

FDA's Center for Biologics Evaluation and Research (CBER) regulates complex biologics such as preventive vaccines for infectious diseases, blood products, and cell and gene therapy products, most of which are for rare diseases. In 2023, CBER approved 18 novel biologics under biologics license applications (BLAs), and of these, 12 were orphan-designated products. These novel biologic approvals include six vaccines, one live biotherapeutic product, seven cell and gene therapy products, and four blood products. This 2023 Demographic Data Summary highlights certain demographic data from the key clinical trial(s) that supported approval of each of these 18 novel biologics. Specifically, the report provides the percentages of clinical trial participants by sex (female), race (White, Black, and Asian), ethnicity (Hispanic) and age (≥ 65 years of age, unless otherwise indicated) for each novel biologic approval.

The demographic information provided for each approval in this summary document can be found in the publicly available package insert, FDA reviews, and other approval documents on FDA's website. The demographic percentages provided for each approval represent the diversity of the study population analyzed for safety, efficacy or both as described in such FDA documents and may not encompass the entire population studied for each product. Additional demographic information may be available in the publicly available FDA documents.

The demographic data provided in this summary is organized in tables by the biological product type categories listed below with footnotes that apply to all four tables at the end.

- **Vaccines**
- **Live Biotherapeutics**
- **Cell and Gene Therapies**
- **Blood Products**

Table 1. Vaccines

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White†	% Black†	% Asian†	% Hispanic	% ≥ 65 years unless otherwise noted
IXCHIQ[S, PI]	Chikungunya Vaccine, Live	Indicated for active immunization for the prevention of disease caused by chikungunya virus in individuals 18 years of age and older who are at increased risk of exposure to chikungunya virus.	4,523	54.7	80.1	14.0	1.9	17.2I	NA
PENBRAYA[S, ST]	Meningococcal Groups A, B, C, W, and Y Vaccine	Indicated for active immunization to prevent invasive disease caused by Neisseria meningitidis serogroups A, B, C, W, and Y. Meningococcal Groups A, B, C, W, and Y Vaccine is approved for use in individuals 10 through 25 years of age.	2412	51.2	78.0	10.2	2.4	25.7	0
ABRYSVO[S, ST]	Respiratory Syncytial Virus Vaccine	Indicated for active immunization for the prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV) in individuals 60 years of age and older	34,284 α	49.2	78.3	12.9	7.8	36.9	100 (≥ 60 years of age)
ABRYSVO[S, CL]	Respiratory Syncytial Virus Vaccine	Indicated for active immunization of pregnant individuals at 32 through 36 weeks gestational age for the prevention of lower respiratory tract disease (LRTD) and severe LRTD caused by respiratory syncytial virus (RSV) in infants from birth through 6 months of age	7357	100	64.5	19.6	12.5	28.9	0

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White[†]	% Black[†]	% Asian[†]	% Hispanic	% ≥ 65 years unless otherwise noted
CYFENDUS[*,S, PI]	Anthrax Vaccine Adsorbed, Adjuvanted	Indicated for post-exposure prophylaxis of disease following suspected or confirmed exposure to Bacillus anthracis in persons 18 through 65 years of age when administered in conjunction with recommended antibacterial drugs.	3,276	57.8	77.9	17.1	1.8	NA	23.8% (51-65 years of age)
AREXVY [S, ST]	Respiratory Syncytial Virus Vaccine, Adjuvanted	Indicated for active immunization for the prevention of lower respiratory tract disease caused by respiratory syncytial virus in individuals 60 years of age and older.	24,966	51.7	79.4	8.7	7.6	5.5	74.5

Table 2. Live Biotherapeutics

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White	% Black	% Asian	% Hispanic	% ≥ 65 years
VOWST[*, S, CL]	fecal microbiota spores, live-brpk	To prevent the recurrence of *Clostridioides difficile* infection (CDI) in individuals 18 years of age and older following antibacterial treatment for recurrent CDI (rCDI).	182	59.9	93.4	4.4	0.5	6.0	56.6

Table 3. Cell and Gene Therapy Products

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White	% Black	% Asian	% Hispanic	% ≥ 65 years unless otherwise noted
CASGEVY*, E, PI	exagamglogene autotemcel (exa-cel)	Indicated for treatment of sickle cell disease (SCD) in patients 12 years of age or older with recurrent vaso-occlusive crises (VOCs).	31	45	3	87	NA	NA	0
LYFGENIA*, S,CLI	lovotibeglogene autotemcel	Indicated for the treatment of patients 12 years of age or older with sickle cell disease and a history of vaso-occlusive events (VOEs).	54	37	NA	88.9	1.9	3.7	0
ROCTAVIAN*, E, CI	valoctocogene roxaparvovec-rvox	Indicated for the treatment of adults with severe hemophilia A (congenital factor VIII deficiency with factor VIII activity <1 IU/dL) without pre-existing antibodies to adeno-associated virus serotype 5 detected by an FDA-approved test.	112	0	69.6	12.5	15.2	4.5	NA
LANTIDRA*,S, ST	donislecel-jujn	Indicated for the treatment of adults with Type 1 diabetes who are unable to approach target HbA1c because of current repeated episodes of severe hypoglycemia despite intensive diabetes management and education.	30	80	100	0	0	3.3	NA
ELEVIDYS*, S, CI	delandistrogene moxeparvovec-rokl	Indicated for treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene.	85	0	76.5	NA	NA	NA	0

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White	% Black	% Asian	% Hispanic	% ≥ 65 years unless otherwise noted
VYJUVEK*, S, E,Cl	beremagene geperpavec-svdt	Indicated for treatment of wounds in patients 6 months of age and older with dystrophic epidermolysis bullosa with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene.	31	35.5	64.5	0	19.4	51.6	0
OMISIRGE*,E, PI	omidubicel-onlv	Indicated for use in adults and pediatric patients 12 years and older with hematologic malignancies who are planned for umbilical cord blood transplantation following myeloablative conditioning to reduce the time to neutrophil recovery and the incidence of infection.	125	42	58	16	14	13	NA$^{\alpha}$

Table 4. Blood Products

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White	% Black	% Asian	% Hispanic	% ≥ 65 years unless otherwise noted
ALYGLO[, S, E, Cl]	immune globulin intravenous, human-stwk	Indicated for treatment of primary humoral immunodeficiency (PI) in adults.	49	42.9	95.9	0	0	8.2	16.32
ADZYNMA[*, S, Cl]	ADAMTS13, recombinant-krhn	Indicated for prophylactic or on demand enzyme replacement therapy (ERT) in adult and pediatric patients with congenital thrombotic thrombocytopenic purpura (cTTP).	48	60.4	64.6	2.1	12.5	2.1	NA
BALFAXAR[*, E, Cl]	concentrate, human-lans prothrombin complex	Indicated for the urgent reversal of acquired coagulation factor deficiency induced by Vitamin K antagonist (VKA, e.g., warfarin) therapy in adult patients with need for an urgent surgery/invasive procedure.	208	43.3	99.5	0	0.5	4.3	74.5 (>60 years)
ALTUVIIIO*[, E, S, ST]	antihemophilic factor (recombinant), Fc-VWF-XTEN fusion protein-ehtl	Indicated for use in adults and children with Hemophilia A (congenital Factor VIII deficiency) for: (1) Routine prophylaxis to reduce the frequency of bleeding episodes; (2) On-demand treatment and control of bleeding	159	0.6	61.0	1.9	18.2	7.7	3.1

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White	% Black	% Asian	% Hispanic	% ≥ 65 years unless otherwise noted
		episodes; and (3) Perioperative management of bleeding.							

Footnotes:
NA – not available
*Orphan-designated drug
[†]The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category.
‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category.
[S] Demographic data are from the population analyzed for safety.
[E] Demographic data are from the population analyzed for efficacy.
[PI] The demographic information provided here can be found in the Package Insert (PI).
[CL] The demographic information provided here can be found in the Clinical Review Memo.
[ST] The demographic information provided here can be found in the Statistical Review Memo.